CIDEBsiRNA Normalizes Liver Fat and Improves Liver Injury Markers in MASLD Patients, as Highlighted at AASLD

November 8, 2026 at 4:00 PM EST

Single administration of CIDEB^{siRNA} resulted in rapid, deep and durable reductions in liver fat by up to 80% that were sustained through 36 weeks

Data from first-in-human trial were shared in oral presentation at AASLD The Liver Meeting® and simultaneously published in *The New England Journal of Medicine*

Regeneron to host virtual "Regeneron Roundtable" investor event to discuss its MASH pipeline programs on Tuesday, November 10 at 8:30 a.m. ET

TARRYTOWN, N.Y., Nov. 08, 2026 (GLOBE NEWSWIRE) -- Regeneron Pharmaceuticals, Inc. (NASDAQ: REGN) today announced the first results from a first-in-human trial that demonstrated a single subcutaneous dose of CIDEB^{siRNA} rapidly normalized liver fat and improved liver injury markers in patients with metabolic dysfunction-associated steatotic liver disease (MASLD) with effects that were sustained through week 36. CIDEB^{siRNA} is an investigational, first-in-class, liver-targeted small interfering RNA (siRNA) therapeutic that mimics the protective effect of rare mutations in the *CIDEB* gene, which were discovered by the Regeneron Genetics Center®. The results were published by *The New England Journal of Medicine* and simultaneously presented in an oral session at the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting® 2026.

"Prior advances in steatotic liver disease have begun to make a difference, but there is so much more progress to be made to help patients normalize liver fat and reverse liver damage," said Jörg Täubel, M.D., F.F.P.M., F.E.S.C., Executive Chairman of Richmond Pharmacology, Visiting Professor at King's College London , Honorary Senior Research Fellow at St. George's University in London, President of The Association for Applied Human Pharmacology and a lead investigator for the trial. "These results show CIDEB^{siRNA} resulted in rapid, deep and sustained reductions in liver fat with improvements in markers of liver injury. With effects out to 36 weeks that support the potential for twice-yearly dosing, alongside the safety profile, the findings highlight CIDEB^{siRNA} as a promising new therapeutic for MASH and other steatotic liver diseases."

MASLD occurs when excess fat accumulates in the liver and encompasses a spectrum of conditions ranging from simple fatty liver to the more serious disease of metabolic dysfunction-associated steatohepatitis (MASH). MASH is a progressive, fibrotic liver disease that affects approximately 13 million U.S. adults, which is characterized by inflammation and cell damage that can lead to cirrhosis, liver transplant, liver cancer and death if not properly managed. Historical clinical trial data report that currently approved MASH therapies have reached mean liver fat reductions of 47-57% at approximately one year.[1,2]

A first-in-human, Phase 1/2 trial evaluated a single subcutaneous dose of CIDEB^{siRNA} at four dose levels (n=30 for CIDEB^{siRNA} groups) versus placebo (n=10) in adults with MASLD. The primary goal of the trial was to evaluate safety and tolerability, with liver fat and markers of liver injury evaluated as secondary endpoints. Meta-analyses of clinical trials of patients with MASH indicate that the depth of liver fat reduction correlates with liver biopsy response (e.g., MASH resolution and fibrosis improvement), supporting changes in liver fat as an informative, early pharmacodynamic readout.

Results for CIDEB^{siRNA} recipients showed:

- **58-80% dose-dependent liver fat reductions at week 36**, which had deepened from the rapid 49-72% reductions at week 6 (as measured by magnetic resonance imaging-proton density fat fraction, MRI-PDFF). Mean decrease in the placebo group was 2.9% at week 36.
- **Liver fat was normalized in a majority (62%) of CIDEB^{siRNA} recipients at week 36** (defined as liver fat content under 5% by MRI-PDFF) versus none in the placebo group. Additionally, 93% had reductions of liver fat of ≥30%, a threshold linked to histological MASH resolution.
- **Improvements were seen in markers of liver injury at early timepoints and sustained through week 36**, with reductions in mean levels of alanine aminotransferase (ALT) across all doses and directionally similar changes in aspartate aminotransferase (AST) levels.

Overall rates of adverse events (AEs) were generally similar between the CIDEB^{siRNA} and placebo groups. All AEs were mild or moderate in severity, except for a severe, serious event of hyperglycemia in a CIDEB^{siRNA}-treated participant that was not attributed to study drug (instead later attributed to a diagnosis of adult-onset Type 1 diabetes). There were no trial discontinuations and no nausea or vomiting AEs reported in the CIDEB^{siRNA} groups, addressing a key tolerability limitation associated with other

therapies.

Longer-term safety and histologic outcomes are being evaluated in multiple, ongoing trials, including Phase 2a, Phase 2b and Phase 3 trials that are now recruiting. Regeneron is also investigating additional drug candidates informed by human genetics for the treatment of steatotic liver diseases.

The potential uses of CIDEB^{siRNA} described above are investigational, and its safety and efficacy have not been evaluated by any regulatory authority.

About the "Regeneron Roundtable" Investor Event
Regeneron will host a virtual investor event to discuss its cardiometabolic and MASH pipeline programs on Tuesday, November 10 at 8:30 a.m. ET. This is the next webcast in an investor event series called the "Regeneron Roundtable," intended to highlight programs from the company's innovative investigational pipeline.

Links to the webcast and to register via telephone may be accessed from the 'Investors and Media' page of Regeneron's website at https://investor.regeneron.com/events-and-presentations. Upon registration, all telephone participants will receive a confirmation email detailing how to join the conference call, including the dial-in number, along with a unique passcode and registrant ID that can be used to access the call. A replay of the conference call and webcast will be archived on the company's website for at least 30 days.

About CIDEB^{siRNA}, the trial and the Regeneron MASLD/MASH Program
CIDEB^{siRNA} is an investigational, first-in-class, liver-targeted siRNA therapeutic that targets a receptor exclusively expressed in the liver to mimic the protective effect of naturally occurring loss-of-function and missense *CIDEB* mutations in a broad population of people with MASLD and MASH.

The ongoing Phase 1/2 randomized, double-blind, placebo-controlled trial is evaluating the safety and tolerability of CIDEB^{siRNA} in adult participants with MASLD and MASH. The primary goal of the trial is to evaluate safety and tolerability with efficacy evaluated as a secondary endpoint through measures such as change in liver fat fraction by MRI-PDFF.

Scientists at the Regeneron Genetics Center® (RGC) analyzed genetic data to discover and/or validate genetic variants in the *CIDEB* and other genes that are associated with liver disease risk. Building on these findings, Regeneron collaborated with Alnylam to generate an siRNA targeting *CIDEB* and has the exclusive rights for the development of and commercialization of CIDEB^{siRNA}; Alnylam receives milestone and royalty payments.

About Regeneron
Regeneron (NASDAQ: REGN) is a leading biotechnology company that invents, develops and commercializes life-transforming medicines for people with serious diseases. Founded and led by physician-scientists, our unique ability to repeatedly and consistently translate science into medicine has led to numerous approved treatments and product candidates in development, most of which were homegrown in our laboratories. Our medicines and pipeline are designed to help patients with eye diseases, allergic and inflammatory diseases, cancer, cardiovascular and metabolic diseases, neurological diseases, hematologic conditions, infectious diseases, and rare diseases.

Regeneron pushes the boundaries of scientific discovery and accelerates drug development using our proprietary technologies, such as *VelociSuite*®, which produces optimized fully human antibodies and new classes of bispecific antibodies. We are shaping the next frontier of medicine with data-powered insights from the Regeneron Genetics Center® and pioneering genetic medicine platforms, enabling us to identify innovative targets and complementary approaches to potentially treat or cure diseases.

For more information, please visit www.Regeneron.com or follow Regeneron on LinkedIn, Instagram, Facebook or X.

Forward-Looking Statements and Use of Digital Media
This press release includes forward-looking statements that involve risks and uncertainties relating to future events and the future performance of Regeneron Pharmaceuticals, Inc. ("Regeneron" or the "Company"), and actual events or results may differ materially from these forward-looking statements. Words such as "anticipate," "expect," "intend," "plan," "believe," "seek," "estimate," variations of such words, and similar expressions are intended to identify such forward-looking statements, although not all forward-looking statements contain these identifying words. These statements concern, and these risks and uncertainties include, among others, the nature, timing, and possible success and therapeutic applications of products marketed or otherwise commercialized by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Products") and product candidates being developed by Regeneron and/or its collaborators or licensees (collectively, "Regeneron's Product Candidates") and research and clinical programs now underway or planned, including without limitation CIDEB^{siRNA} (also known as ALN-CIDEB); the likelihood, timing, and scope of possible regulatory approval and commercial launch of Regeneron's Product Candidates and new indications for Regeneron's Products, such as CIDEB^{siRNA} for the treatment of metabolic dysfunction-associated steatotic liver disease as discussed in this press release as well as the other clinical programs for the treatment of metabolic dysfunction-associated steatohepatitis discussed or referenced in this press release; uncertainty of the utilization, market acceptance, and/or commercial success of Regeneron's Products and Regeneron's Product Candidates and the impact of studies (whether conducted by Regeneron or others and whether mandated or voluntary), including the studies discussed or referenced in this press release, on any of the foregoing or any potential regulatory approval of Regeneron's Products and Regeneron's Product Candidates (such

as CIDEB^{siRNA}); the ability of Regeneron's collaborators, licensees, suppliers, or other third parties (as applicable) to perform manufacturing, filling, finishing, packaging, labeling, distribution, and other steps related to Regeneron's Products and Regeneron's Product Candidates; the ability of Regeneron to manage supply chains for multiple products and product candidates and risks associated with tariffs and other trade restrictions; safety issues resulting from the administration of Regeneron's Products and Regeneron's Product Candidates (such as CIDEB^{siRNA}) in patients, including serious complications or side effects in connection with the use of Regeneron's Products and Regeneron's Product Candidates in clinical trials; determinations by regulatory and administrative governmental authorities which may delay or restrict Regeneron's ability to continue to develop or commercialize Regeneron's Products and Regeneron's Product Candidates; ongoing regulatory obligations and oversight impacting Regeneron's Products, research and clinical programs, and business, including those relating to patient privacy; the availability and extent of reimbursement or copay assistance for Regeneron's Products from third-party payors and other third parties, including private payor healthcare and insurance programs, health maintenance organizations, pharmacy benefit management companies, and government programs such as Medicare and Medicaid; coverage and reimbursement determinations by such payors and other third parties and new policies and procedures adopted by such payors and other third parties; changes to drug pricing regulations and requirements and Regeneron's pricing strategy, including in connection with Regeneron's April 2026 agreements with the U.S. government; other changes in laws, regulations, and policies affecting the healthcare industry; competing products and product candidates (including biosimilar products) that may be superior to, or more cost effective than, Regeneron's Products and Regeneron's Product Candidates; the extent to which the results from the research and development programs conducted by Regeneron and/or its collaborators or licensees may be replicated in other studies and/or lead to advancement of product candidates to clinical trials, therapeutic applications, or regulatory approval; unanticipated expenses; the costs of developing, producing, and selling products; the ability of Regeneron to meet any of its financial projections or guidance and changes to the assumptions underlying those projections or guidance; the potential for any license, collaboration, or supply agreement, including Regeneron's agreements with Sanofi and Bayer (or their respective affiliated companies, as applicable) as well as Regeneron's license agreement with Alnylam Pharmaceuticals, Inc. referenced in this press release, to be cancelled or terminated; the impact of public health outbreaks, epidemics, or pandemics on Regeneron's business; and risks associated with litigation and other proceedings and government investigations relating to the Company and/or its operations (including the pending civil proceedings initiated or joined by the U.S. Department of Justice and the U.S. Attorney's Office for the District of Massachusetts), risks associated with intellectual property of the Company or other parties and pending or future litigation or patent office proceedings relating thereto (including without limitation patent litigation and other related proceedings relating to Regeneron's Products), the ultimate outcome of any such proceedings and investigations, and the impact any of the foregoing may have on Regeneron's business, prospects, operating results, and financial condition. A more complete description of these and other material risks can be found in Regeneron's filings with the U.S. Securities and Exchange Commission, including its Form 10-K for the year ended December 31, 2025 and its Form 10-Q for the quarterly period ended September 30, 2026. Any forward-looking statements are made based on management's current beliefs and judgment, and the reader is cautioned not to rely on any forward-looking statements made by Regeneron. Regeneron does not undertake any obligation to update (publicly or otherwise) any forward-looking statement, including without limitation any financial projection or guidance, whether as a result of new information, future events, or otherwise.

Regeneron uses its media and investor relations website and social media outlets to publish important information about the Company, including information that may be deemed material to investors. Financial and other information about Regeneron is routinely posted and is accessible on Regeneron's media and investor relations website (https://investor.regeneron.com) and its LinkedIn page (https://www.linkedin.com/company/regeneron-pharmaceuticals).

[1] Harrison SA, Bedossa P, Guy CD, et al. A Phase 3, Randomized, Controlled Trial of Resmetirom in NASH with Liver Fibrosis. *N Engl J Med*. 2024;390(6):497-509. doi:10.1056/NEJMoa2309000.

[2] Flint A, Andersen G, Hockings P, et al. Randomised clinical trial: semaglutide versus placebo reduced liver steatosis but not liver stiffness in subjects with non-alcoholic fatty liver disease assessed by magnetic resonance imaging. *Aliment Pharmacol Ther*. 2021;54(9):1150-1161. doi:10.1111/apt.16608.

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